

Stem Cell Agency Spinal Cord Injury Clinical Trial Passes Safety Hurdles

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Oakland, CA – A clinical trial using stem cells to treat people with recent spinal cord injuries has cleared two key safety hurdles, and been given approval to expand the therapy to a larger group of patients with a much higher dose of cells.

Asterias Biotherapeutics announced that its Data Monitoring Committee (DMC) has reviewed the safety data from the first two groups of patients treated and found no problems or adverse side effects. One group of three patients was given 2 million cells. The second group of five patients received 10 million cells. Asterias is now cleared to enroll another 5-8 patients with 20 million cells.

The SciStar study, funded in part by the California Institute for Regenerative Medicine (CIRM) is a Phase 1/2a clinical trial that is designed to test first the safety and then the effectiveness of Asterias' AST-OPC1 cells. These are a form of cells called oligodendrocyte progenitors, which are capable of becoming several different kinds of cells some of which play a supporting role and help protect nerve cells in the central nervous system, including areas of the spinal cord that are damaged in spinal cord injury.

"Our focus is always on the patient, so making sure a potential therapy is safe is an important first step," says C. Randal Mills, Ph.D., the President and CEO of CIRM. "I recently met with Jake Javier, a young man who was treated in this trial, and heard first-hand what he and his family are going through in the aftermath of his injury. But I also saw a young man with remarkable courage and determination. It is because of Jake, and the others who volunteer to take part in clinical trials, that progress is possible. They are true heroes."

The patients enrolled in the trial have experienced injuries in the C5-C7 vertebrae and have essentially lost all feeling and movement below the injury site, with severe paralysis of the upper and lower limbs. They are treated with the cells 14 to 30 days after the injury was sustained.

"The positive safety data in the previous phase 1 study and in the ongoing phase 1/2a study gives us the confidence to now proceed to administration of 20 million cells, which based on our significant pre-clinical research is likely well within the dosing range where we would expect to see clinically meaningful improvement in these patients," said Dr. Edward Wirth, Chief Medical Officer of Asterias Biotherapeutics.

About CIRM

At CIRM, we never forget that we were created by the people of California to accelerate stem cell treatments to patients with unmet medical needs, and act with a sense of urgency to succeed in that mission.

To meet this challenge, our team of highly trained and experienced professionals actively partners with both academia and industry in a hands-on, entrepreneurial environment to fast track the development of today's most promising stem cell technologies.

With \$3 billion in funding and approximately 300 active stem cell programs in our portfolio, CIRM is the world's largest institution dedicated to helping people by bringing the future of cellular medicine closer to reality.

For more information, go to www.cirm.ca.gov